This presentation contains forward-looking statements relating to: guidance regarding anticipated financial results for 2019; ambition to continue to deliver double-digit revenue growth (and strong financials); expected growing product portfolio; ambition to convert patients to Ultomiris in the future for PNH (and aHUS); anticipated future product launches (including future subcutaneous products); plans for future clinical programs and the expansion of those programs (and expanding the Company’s product pipeline and the means expected to achieve such expansion); the nature, timing, and possible success and therapeutic applications of Alexion’s products, product candidates, and research and clinical programs now underway or planned, including without limitation Soliris, Ultomiris, ALN-X1101, CAEL-101, ALN-X1830, ALN-X1720 and ABY-039 (and subcutaneous formulations of certain products and product candidates); the anticipated timing for the initiation and completion of clinical trials and product development; the long-term durability of current and future products; Alexion’s earlier stage product candidates; the extent to which the results from research programs or preclinical testing may lead to advancement of product candidates to clinical trials or therapeutic applications; the likelihood and timing of regulatory filings, possible regulatory approval and commercial launch of Alexion product candidates and new indications for marketed products (including 10 potential launches over the next five years); the potential success of product development programs (including anticipated timelines for potential future first-in-class-and/or first-to-market; Alexion is significantly increasing addressable patient populations (and the product candidates that could lead to treatment for such patients); the expected benefits of the Four Pillars of growth (and possible new pillars of growth); the Company is building a dynamic, blockbuster franchise in PNH/aHUS, metabolic, neurology and FcRn; the potential of the therapeutic benefits for our products and product candidates (including the potential to be transformative therapies for certain indications); future growth trajectories of our products and product candidates (including Strensiq, Kanuma and treatments for patients with gMG); potential for certain Alexion products to become the standard of care for certain indications; timing of biosimilar entry and that the portion of the business at risk to biosimilars is minimal; future anticipated pricing strategies for certain products (and potential discounts relative to other indications) and potential cost-savings; expected acceleration of neuromuscular growth with gMG and other potential neurological indications; ambition for gMG patients to comprise largest Soliris treated patient population; anticipated patient preferences for certain products, dosing schedules and subcutaneous delivery mechanisms; expected increase to Soliris/Ultomiris patient base: goal of providing treatment options for entire spectrum of gMG patients (and Alexion’s expanding gMG portfolio strategy and path to category leadership); potential for Soliris to be the first approved therapy for NMOSS; certain products in clinical trials (for ALS and PPMs) are high risk/high reward but have the potential to be transformative for patients; neurology franchise represents a multi-billion dollar revenue potential; potential of FcRn assets as therapy for neurological conditions; certain initiatives may result in identification of more patients for certain of our products and product candidates and earlier diagnosis of disease states; ALN-X1830 and ALN-X1840 have potential to be first line treatment utilization for applicable indications; building a potential leading FcRn portfolio (with multiple opportunities to address a range of IgG mediated diseases); there will be multiple INDs over the coming years; plan to develop ALN-1720 for multiple new indications and therapeutic areas; and Alexion’s 2019 objectives. Forward-looking statements are subject to factors that may cause Alexion’s results and plans to differ materially from those forward-looking statements, including for example: our dependence on sales from our principal product (SOLIRIS); our ability to fully realize the benefits from the commercialization of our products; the profitability of our products; the acceptance of our products by payors and patients; the continued retention of key employees; successful qualification and acceptance of our products for reimbursement by governmental authorities; the timely receipt of regulatory approvals for new products and product candidates, and the continued approval of existing products under existing regulatory conditions; the expected benefits related to the anticipated use of Ultomiris; the achievement of planned financial and operating results; the success of Ultomiris and the satisfaction of the conditions precedent to the ultimate commercialization of our product candidates; our estimates related to timing, cost and results of expansion programs; the continued acceptance of our products and any anticipated new products; the effects of competition; uncertainties in the timing or successful completion of research and development programs and clinical trials and the success and cost of obtaining regulatory approvals; our ability to attract and retain qualified scientific, clinical and sales personnel; difficulties in obtaining adequate coverage and reimbursement; the effects of healthcare reform; the potential extent of patent protection for our products; unexpected delays in manufacturing and supply; the adequacy of our legal and executive officers; the impact of emerging technologies; the effect of natural disasters, earthquake, terrorism, war and political unrest; international trade and currency controls; our ability to successfully pursue strategic initiatives; the effect of changes in foreign, federal, state and local laws and regulations; our ability to accurately estimate the potential size of the market and to achieve market acceptance; the impact of product recalls; the potential for product liability claims; the impact of pandemics or other public health concerns; deterrence of potential purchasers; the timing of the potential completion of our current development efforts; the impact of competition and consolidation in the healthcare industry; and our ability to obtain adequate financing; and other risks and uncertainties that should be read in conjunction with the statements under “Item 1A. Risk Factors” and elsewhere in this Form 10-K and in our other filings with the SEC. Alexion disclaims any obligation to update any of these forward-looking statements to reflect events or circumstances that arise after the date hereof, except when a duty arises under law.

In addition to financial information prepared in accordance with GAAP, this presentation also contains non-GAAP financial measures that Alexion believes, when considered together with the GAAP information, provide investors and management with supplemental information relating to performance, trends and prospects that provide a more complete understanding of our operating results and financial position during different periods. The non-GAAP amounts exclude the impact of the following GAAP items: share-based compensation expense, fair value adjustment of inventory acquired, amortization of purchased intangible assets, changes in fair value of contingent consideration, restructuring and related expenses, upfront payments related to licenses and collaborations, acquired in-process research and development assets, impairment of intangible assets, gain in value of strategic equity investments, litigation charges, gain or loss on sale of a business and certain adjustments to income tax expense. These non-GAAP financial measures are not intended to be considered in isolation or as a substitute for, or superior to, the financial measures prepared and presented in accordance with GAAP, and should be reviewed in conjunction with the relevant GAAP financial measures. Please refer to the Alexion website for GAAP to non-GAAP 2019 Financial Guidance for explanations of the amounts adjusted to arrive at non-GAAP operating margin and non-GAAP earnings per share amounts for the projected twelve months ending December 31, 2019.
Our Mission
Serving patients and their families is our unwavering mission – they are our guiding star and they inspire us to continue to find answers.
We act with integrity, urgency, and discipline because we know that lives are at stake.
**OUR FOCUS IS IN RARE DISEASE**

>7,000 rare diseases identified

Only 500 rare diseases have approved therapies

~30M patients diagnosed in US, 50% are children

Four transformative therapies across five rare diseases in our growing portfolio

- **Paroxysmal Nocturnal Hemoglobinuria (PNH)**

  Prior to SOLIRIS®, up to 35% of patients with available support did not survive beyond 5 years

- **Atypical Hemolytic Uremic Syndrome (aHUS)**

  Prior to SOLIRIS®, 79% of patients died, required kidney dialysis, or had permanent kidney damage within three years of diagnosis

- **Generalized Myasthenia Gravis (gMG)**

  Debilitating, chronic and progressive autoimmune neuromuscular disease that can lead to inability to walk, talk, eat or breathe

- **Hypophosphatasia (HPP)**

  Prior to STRENSIQ®, children with symptoms prior to 6 months of age had 73% mortality rate at 5 years

- **Lysosomal Acid Lipase Deficiency (LAL-D)**

  Prior to KANUMA®, Median age of death was 3.7 months in infants with rapid disease progression

Alexion’s transformation began in 2017

Culture Team Appointed: 34 leaders, >350 employees engaged in defining our aspirational culture

June 2017
Roll-out our redefined Cultural Values

July 2017
Anne-Marie Law appointed Chief HR Officer

February 2018
Ellen Chinaiara, J.D. appointed General Counsel

October 2017
SOLIRIS® approved in US with broad label for gMG

Late 2017 - Early 2018
Alexion Board of Directors New Refresh Completed

September 2018
Alexion acquires Syntimmune, including ALXN1830 (SYNT001)

September 2018
Positive SOLIRIS® Phase 3 data in NMOSD

October 2018
Announced Dicemia Collaboration

February 2019
Granted Priority Review by FDA for NMOSD PDUFA June 28th

January 2019
Announced Caelum Collaboration

2017

March 2017
Ludwig Hantson, PhD appointed Chief Executive Officer

April 2017
Rana Stallias appointed SVP, Global Communications & Culture

July 2017
Paul Clancy appointed Chief Financial Officer

December 2017
Announced Halozyme collaboration

April 2018
Alexion acquires Wilson Therapeutics, including ALXN1840 (WTX101)

Summer 2018
Alexion opens new corporate headquarters in Boston

March 2018
Announced Positive Phase 3 ULOTRIS® PNH 301 Study Results

June 2018
Announced Complement Pharma Collaboration

April 2018
Announced Positive Phase 3 ULOTRIS® PNH 302 Study Results

June 2018
Announced Positive Phase 3 ULOTRIS® aHUS Study Results

October 2018
SOLIRIS® gMG launch officially best Alexion launch to date

March 2019
Announced Attribu Collaboration

March 2019
Announced Zealad Collaboration

January 2019
Aradhana Sarin, MD appointed Chief Strategy & Business Officer

Anne-Marie Law appointed Chief Patient and Employee Experience Officer

January 2019
Announced Attribu Collaboration

2018

2019
ALEXION’S NEXT CHAPTER

Transforming Compliance, Culture & Talent

Delivering Strong Financials

Executing on Our Refocused Strategy
FOCUS ON CULTURE, COMPLIANCE & TALENT

We have strengthened and refreshed our employee base:

- Reinforced Culture of Compliance
- Strengthened Leadership Team
- Refreshed Board of Directors
- Restructured to Optimize Organization and Resource Allocation
- Relocated Corporate HQ to Boston

Hired and on-boarded ~30% of our total employee base in the past year*

Leading with Diversity: >50% women on the Executive Team and in senior leadership positions

*While reducing total headcount since 2016
Financial Ambition to Continue to Deliver Double-Digit Revenue and Non-GAAP EPS Growth

*Mid-point of 2019 Guidance range provided February 4, 2019
Reconciliations of our GAAP to non-GAAP financial results are available at www.alexion.com
Extending durability of our legacy business in PNH and atypical HUS

- **ULTOMIRIS® Phase 3 PNH**
- **ULTOMIRIS® Phase 3 atypical HUS**
- **ULTOMIRIS® SubQ**
- **ULTOMIRIS® PNH launch**
- **ULTOMIRIS® atypical HUS filing**

**Potential Redefined Standard of Care in PNH & atypical HUS**
- Ambition to convert >70% patients to ULTOMIRIS within 2 years of launch
- ULTOMIRIS first-in-class C5 SubQ launched

**Vision & Strategy**
- **Ludwig Hansson, Ph.D., CEO**

**Timeline**
- **2017**
- **2019**
- **2021**

**Clinical Programs**
- **4 clinical programs**
- **16 clinical programs**
- **Ambition to continue to expand development programs**

**At least 6 programs in Ph 3 in 2020**

**Significant pipeline progress**
<table>
<thead>
<tr>
<th>Preclinical</th>
<th>Early Clinical</th>
<th>Advanced Clinical</th>
<th>Registration</th>
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<tbody>
<tr>
<td>Internal Complement Programs</td>
<td>ULTOMIRIS QW SubQ</td>
<td>ULTOMIRIS IV PNH</td>
<td>SOLIRIS® IV NMOSD</td>
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**Hematology & Nephrology**

**Neurology**
<table>
<thead>
<tr>
<th>Preclinical</th>
<th>Early Clinical</th>
<th>Advanced Clinical</th>
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<tr>
<td>Internal Complement Programs</td>
<td>ALXN1810 SubQ (ULTOMIRIS/PH20)</td>
<td>ULTOMIRIS QW SubQ</td>
<td>SOLIRIS® NMOSD</td>
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<td>Complement Pharma</td>
<td>CAEL-101 AL Amyloidosis</td>
<td>ULTOMIRIS High Concentration</td>
<td>ULTOMIRIS® aHUS</td>
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<td>Dicerna</td>
<td>ALXN1830 (SYNT001) WAIHA</td>
<td>ALXN1830 (SYNT001) gMG</td>
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<td>Zealand</td>
<td>ABY-039 Rare Autoimmune</td>
<td>ALXN1840 (WTX101) Wilson Disease</td>
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<td>Next-Gen Treatment for HPP</td>
<td>ALXN1820 (Anti-C5 Bi-specific)</td>
<td>ALXN1830 (SYNT001) WAIHA</td>
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<tr>
<th>Hematology &amp; Nephrology</th>
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<tr>
<td>Metabolics</td>
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<tr>
<td>Neurology</td>
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<tr>
<td>FcRn</td>
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<tr>
<td>TBD</td>
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*Italicized* = plan to initiate in 2019
Diversifying the Alexion Portfolio

1. Hematology & Nephrology
2. Neurology
3. Metabolics
4. FcRn Portfolio

Financial Ambition to Continue to Deliver Double-Digit Revenue and Non-GAAP EPS Growth
LONG-TERM DURABILITY IN PNH & ATYPICAL HUS*

Continued underlying high single-digit volume growth

Transformational treatments address critical disease measures

Compelling value proposition for all stakeholders

Best-in-class Conversion Target

>70%

Within 2 years of launch

Hematology & Nephrology

*ULTOMIRIS is currently not approved for the treatment of aHUS
SOLIRIS® gMG is best Alexion launch to date

SOLIRIS® NMOSD PDUFA date of June 28, 2019; Priority Review granted

Leverage commercial capabilities of newly expanded, dedicated Neurology salesforce

ULTOMIRIS® development programs in four Neurology indications; ALXN1830 in development for gMG
Wilson disease poorly managed by current treatments, low compliance and potential for neurological worsening

1\textsuperscript{st} Line Chelation: Penicillamine
2\textsuperscript{nd} Line Chelation: Trientine
Maintenance: Zinc

Addressable Population
Prevalence 1 in 30,000
~10K in EU5 and ~10K in US
~50% Diagnosed and Treated
~5K in EU5 and ~5K in US

Differentiated product profile compared to currently available treatments

ALXN1840 (WTX101)
10,000-fold higher affinity for Cu
Rapid onset of action
Specifically binds to Cu
Safe Cu transport in blood
Oral, once-daily dosing
Natural Cu excretion through bile

Ongoing Phase 3 Trial Powered for Superiority
BUILDING A COMPELLING FcRn PORTFOLIO

WAIHA
No Effect on Albumin
Rapid Onset of Action

ALXN1830 Syntimmune

FCRH

Potential Best-in-Class SubQ
Long Half-life
Low Molecular Weight

Potent Binding Affinity

gMG

ABY-039 Affibody

No Effect on Albumin
Rapid Onset of Action
SIGNIFICANTLY EXPANDING OUR ADDRESSABLE PATIENT POPULATIONS

Aligned with our strategy to move from a focus on ultra-rare to rare diseases

- Continuing to expand addressable patient populations with SOLIRIS® and ULTOMIRIS®
- Accelerating neurology growth in gMG and other potential neurological indications
- FcRn inhibitors have potential to become a new generation of therapeutics for IgG mediated diseases
- Significant opportunity in Wilson Disease and AL Amyloidosis

VISION & STRATEGY | LUDWIG HANTSON, PHD, CEO

Diagnosed Prevalence (000's)

- PNH
- atypical HUS
- hPP
- LAL-D
- NMOSD
- gMG
- Refractory gMG
- Wilson Disease
- AL Amyloidosis
- WAIHA
- ALS
- PPMS

~10K Total Patients

~230K Total Patients

~10K Total Patients
BUILDING A PORTFOLIO WITH DURABLE GROWTH

Organic Clinical Pipeline

Business Development

Internal Complement Research

Hematology & Nephrology

Neurology

Metabolics

FcRn Portfolio

VISION & STRATEGY | LUDWIG HANTSON, PHD, CEO

RARE INSPIRATION. CHANGING LIVES.
DAVID R. BRENNAN, CHAIRMAN

- Appointed Chairman of the Board in 2017
- Served as Director of Alexion since July 2014
- Served as Interim CEO of Alexion from December 2016 to March 2017
- Prior to joining Alexion’s Board of Directors, served as CEO and Executive Director of AstraZeneca PLC
EMBARKING ON ALEXION’S NEXT CHAPTER

Delivering on Our Strategy

☑️ Management & Board refresh
☑️ Transformed culture, values, talent
☑️ SOLIRIS® gMG best Alexion U.S. launch
☑️ Positive ULTOMIRIS® data in PNH and aHUS
☑️ Early PNH U.S. approval; Promising initial conversion
☑️ Remarkable pivotal NMOSD data
☑️ Internal pipeline refresh and progress
☑️ BD execution: 4 clinical, 3 pre-clin deals in 12 months
☑️ Delivering on Financials

Over 10 Potential Launches Over Next Five years

- 2019
  - SOLIRIS NMOSD
  - ULTOMIRIS PNH

- 2020
  - ULTOMIRIS aHUS

- 2021
  - ULTOMIRIS QW SubQ
  - PNH, aHUS

- 2022
  - ULTOMIRIS IV gMG
  - ULTOMIRIS SubQ gMG
  - ALXN1840 Wilson
  - CAEL101 AL Amyloidosis

- 2023
  - ALXN1830 gMG
  - ALXN1830 WAIHA
  - ULTOMIRIS IV NMOSD
  - ULTOMIRIS SubQ NMOSD
Diversifying the Alexion Portfolio

1. Hematology & Nephrology
2. Neurology
3. Metabolics
4. FcRn Portfolio

Financial Ambition to Continue to Deliver Double-Digit Revenue and Non-GAAP EPS Growth
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<th>Time</th>
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<tr>
<td>8:30am</td>
<td>VISION &amp; STRATEGY</td>
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<tr>
<td>9:00am</td>
<td>HEMATOLOGY / NEPHROLOGY</td>
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<td></td>
<td>Durability in our legacy business</td>
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<td>PNH: KOL Perspective</td>
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<tr>
<td>9:40 am</td>
<td>NEUROLOGY</td>
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<td>Continued growth &amp; opportunity to expand</td>
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<td>NMOSD: KOL Perspective</td>
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<td>10:20 am</td>
<td>Q&amp;A SESSION 1 / Break</td>
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<td>10:50 am</td>
<td>METABOLICS</td>
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<td>Transforming liver &amp; bone disease</td>
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<td></td>
<td>WILSON DISEASE: KOL Perspective</td>
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<td>11:20 am</td>
<td>FcRn PORTFOLIO</td>
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<td>Future of Rare Autoimmune Diseases</td>
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<td>INTERNAL RESEARCH</td>
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<td>Complement Expertise</td>
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<td>Q&amp;A SESSION 2 &amp; Lunch</td>
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HEMATOLOGY & NEPHROLOGY
Brian Goff, Chief Commercial Officer
John Orloff, M.D., Head of R&D
Diversifying the Alexion Portfolio

1. Hematology & Nephrology
2. Neurology
3. Metabolic
4. FcRn Portfolio

Financial Ambition to Continue to Deliver Double-Digit Revenue and Non-GAAP EPS Growth
## SOLIRIS®: Established a Legacy

- In the U.S. in 2007 SOLIRIS® was the first FDA approved treatment for PNH.
- In the U.S. in 2011 SOLIRIS® was the first FDA approved treatment for aHUS.
- 35% of PNH patients died within 5 years despite supportive care.
- 79% of aHUS* patients died within 3 years of diagnosis.
- As of 2Q2017, over 2,500 PNH and aHUS patients were treated with SOLIRIS® in the United States.

### Historically High Mortality

### 2,500

### ULTOMIRIS®: Potential to be the new Standard of Care

- ULTOMIRIS was approved by FDA on December 21, 2018 for the treatment of PNH.
- Patients can safely switch from SOLIRIS® to ULTOMIRIS.
- Rapid, complete, sustained complement inhibition.
- Every 8 week IV dosing profile.
- Globally sustainable pricing strategy.
- Best-in-class facilitated conversion target of ≥70% of PNH patients in two years post-launch.
- Similar ambition in aHUS.

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*ULTOMIRIS is currently not approved for the treatment of aHUS*
ULTOMIRIS® VALUE PROPOSITION IN PNH

Efficacy
- Efficacy on three clinically meaningful disease measures:
  - Majority achieved LDH normalization
  - Increased rates of transfusion avoidance
  - Low-breakthrough hemolysis rates

Safety
- Safety demonstrated in largest-ever Phase 3 PNH clinical program
- Comprehensive safety data supporting safe switch from SOLIRIS®; both target the same C5 epitope; no wash-out period
- Strong safety backbone with SOLIRIS® on market for over a decade

Delivery
- Fewer infusion clinic visits
- Q8W IV minimizes treatment burden
- Rapid, complete, sustained complement inhibition
- Patient-friendly IV and potential subQ treatment options

Access
- Globally sustainable pricing strategy
- Additional savings from fewer annual infusions, reduced incidence of breakthrough hemolysis and increased work productivity
- Phase 3 program included broad patient population; reflective of real-world setting

More than a decade of experience and trust built upon SOLIRIS® foundation
In Study 301 and 302: No Patients in the ULTOMIRIS Treatment Groups Experienced Elevated Serum Free C5 Concentrations (≥0.5 µg/mL)

ULTOMIRIS Demonstrates Reduced Incidence of Breakthrough Hemolysis

Rate of Breakthrough Hemolysis

- 10.7%
- 4.0%*
- 5.1%
- 0.0%

*ULTOMIRIS breakthrough hemolysis rate not C5 related

>440 patients treated in Phase 3 and >650 patient years of exposure across program
REGIS PEFFAULT DE LATOUR, MD, PhD

Head of the Reference Center for Aplastic Anemia and PNH, Saint-Louis Hospital, Paris

• KOL Perspective: The Role and Differentiation of Intravascular (IVH), Extravascular (EVH), and Breakthrough (BTH) Hemolysis in PNH
PNH: A LIFE THREATENING ULTRA-RARE DISEASE

PAROXYSMAL NOCTURNAL HEMOGLOBINURIA

is a chronic, debilitating, and potentially life threatening ultra-rare blood disorder, with an average age of onset in the early 30s\(^1\-^3\)

BEFORE SOLIRIS\textregistered, \(\frac{1}{3}\) OF PATIENTS DIED WITHIN 5 YEARS OF DIAGNOSIS\(^2\)

SOME SYMPTOMS OF PNH\(^4\-^8\)

- Anemia
- Fatigue
- Dark urine
- Difficulty swallowing
- Abdominal pain
- Shortness of breath
- Formation of blood clots (thrombosis) that could lead to premature death

Increased Destruction of RBCs

Due to complement-mediated destruction of RBCs

Decreased Production of RBCs

BMF

Two-thirds may have AA or MDS

Ravulizumab addresses BTH associated with elevations in free C5 in patients with PNH

Efficacy of ravulizumab is noninferior to eculizumab and safety profile of ravulizumab is similar to that of eculizumab

AA, aplastic anemia; BMF, bone marrow failure; BTH, breakthrough hemolysis; C5, complement component 5; C3, complement component 3; EVM, extravascular hemolysis; IVH, intravascular hemolysis; LDH, lactate dehydrogenase; MDS, myelodysplastic syndrome; PNH, paroxysmal nocturnal hemoglobinuria; RBC, red blood cell.

3. ULTOMIRIS® (ravulizumab-cwvz) [Prescribing Information]. Alexion Pharmaceuticals, Inc; December 2018.
Hematological Response to Eculizumab

- **Hgb ≥11**: 36.6% (n=15)
  - 12.2% clinically evident EVH
- **8 ≤ Hgb <11**: 43.9% (n=18)
  - 7.3% due to underlying AA
- **Hgb <8**: 17% (n=3)

**Optimal Responders**: Patients achieving transfusion independence with hemoglobin levels ≥11 g/dL

**Major Responders**: Patients achieving transfusion independence with hemoglobin levels ≥8 g/dL

**Partial Responders**: Reduction of transfusion requirement by >50% without abrogation of blood transfusions

- 3/5 (~7%) patients experienced clinically evident C3-mediated EVH

**Minor Responders**: No significant change in blood transfusion requirement (reduction ≤50%) or hemoglobin levels but with marked reduction of LDH levels

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C3-mediated EVH only clinically evident in ~7% of patients

- Hb, hemoglobin.
- Over 80% (33/41) of patients were transfusion-independent on eculizumab. ~17% (7/41) of patients had breakthrough IVH. All minor responders in this cohort were attributed to underlying AA.
- Partial responders included 5/41 (12%) patients. Patient #11 had breakthrough hemolysis due to IVH, and patient #38 had 5% C3 deposition, which was less than the median C3 deposition in the optimal responder group (~20% C3 deposition in this group). Therefore, clinically significant EVH in this cohort was found in 3/41 patients (7%), possibly also a result of underlying mild to moderate AA in these patients.
PNH is a life-threatening disease characterized by IVH\(^1,2\)

SOLIRIS\(^\text{®}\) & ULTOMIRIS\(^\text{®}\) inhibit C5, the cause of complement-mediated IVH; patients with PNH on SOLIRIS\(^\text{®}\) who remain dependent on transfusions may be experiencing BTH or have a concurrent condition such as BMF and/or EVH\(^2-4\)

ULTOMIRIS addresses BTH related to inadequate C5 inhibition, a key unmet need in patients with PNH receiving SOLIRIS\(^\text{®}\)\(^5-7\)
At what Hemoglobin levels would you consider a patient in need of transfusion?
What has been your experience treating PNH patients with ULTOMIRIS®?
How do PNH Patients feel about a less frequent IV treatment?

All but one patient out of >450 elected to continue on treatment with ULTOMIRIS in the extension study.

93% of PNH patients surveyed preferred ULTOMIRIS over SOLIRIS®.

“…assuming it works exactly as effectively as SOLIRIS®…less frequent infusions would be my ideal new treatment to try.”

“…getting infusions every 2 weeks is very difficult and I don’t have home infusions… I have to go to a facility, hospital or infusion center. So I would love to have access to a drug that had much fewer infusions.”

1: Internal Alexion data from ALXN1210-PNH-302 extension study
ULTOMIRIS Launch in PNH Lays Foundation for Durability and Leadership

- Best-in-class conversion goal of >70% in first two years following launch
- Compelling value proposition with strength of data, ease of switch and pricing strategy
**ULTOMIRIS® Best-in-Class Product Profile**

- LDH Normalization
- Transfusion avoidance
- Low-breakthrough hemolysis rates
- Comprehensive data supporting seamless, safe switch from SOLIRIS®
- Patient-friendly IV and Potential SubQ treatment options
- Fewer infusion clinic visits
- Comprehensive safety profile
Nearly all commercial lives have access; enabling conversion to or naïve initiation of ULTOMIRIS

~1/3 of commercial lives have a specific ULTOMIRIS coverage policy in place

Planning underway for launches in Germany and Japan in 2019
Established globally sustainable pricing strategy

Atypical HUS and higher-volume indications (gMG & NMOSD)¹ expected to realize greater discount compared with PNH
- ~30% discount to annual SOLIRIS® maintenance dosing

Consistent with our strategy to shift our focus from ultra-rare to rare diseases

*Loading dose in Yr1 accounts for the 10% premium to labeled SOLIRIS® PNH maintenance dose
¹) ULTOMIRIS is not yet approved in aHUS, gMG, and NMOSD
**Clinical Data**

- **Components of Primary Endpoint**
  - Platelet count normalization: 83.9%
  - LDH normalization: 76.8%
  - Increase in renal function*: 58.9%

**Impact on TMA**

- **TMA Manifestation Rate**
  - Off SOLIRIS® Treatment (n=39): 1.0
  - On SOLIRIS® Treatment (n=76): 15.6

- **TMA Event Rate** per 100 Patient-Years:
  - Off SOLIRIS® Treatment: ~3x
  - On SOLIRIS® Treatment

- **Safety consistent with that observed in 301 and 302 Phase 3 PNH trials**

**Impact on TMA**

- Often there is poor persistence due to frequent dosing for SOLIRIS®, “silent” symptomology, and cost
- Risk of TMA is higher when patients are off treatment
- Opportunity to improve compliance, helping to reduce risk of life-threatening TMA, with ULTOMIRIS® Q8W dosing and value proposition

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*ULTOMIRIS is currently not approved for the treatment of aHUS*
Program Overview

- Potential approval end of 2021
  - Potential approval in PNH & atypical HUS with current Phase 3 design
  - 100mg/mL subQ formulation
- Ongoing Phase 3 in PNH patient population, allows for approval in atypical HUS pending positive results

### Randomized, Multicenter Phase 3 Program

- **Control arm:** ULTOMIRIS IV Q8W
- **Active Arm:** ULTOMIRIS SubQ QW

- 4-week Screening Period
- 10-Week Treatment Period
- Extension Period to 1 Year

---

West’s SmartDose® drug delivery platform

- **Potential first to market** SubQ option for PNH & atypical HUS
- **Previously approved by the FDA** for use with another combination product
- **Patient friendly device** with ~10 min total infusion time, no visible needle, no need for reconstitution

1) West and the diamond logo, SmartDose® and logo, and the external product configuration of West’s SmartDose® drug delivery platform are the intellectual property of West Pharmaceutical Services, Inc. or one of its subsidiaries, in the United States and other countries.
Remove devices from packaging (7mL dose requires two 3.5mL devices)

Insert drug cartridge

Apply directly to skin

LED status indicator will blink green and emit gentle hum while dose is administered

Light will show solid green once dose is complete

Remove devices from body and safely dispose

Reliable

No Visible Needle

Familiar

No Reconstitution

1) SmartDose® and the external product configuration of West's SmartDose® drug delivery platform are the intellectual property of West Pharmaceutical Services, Inc. or one of its subsidiaries, in the United States and other countries.
Evolution of Our PNH & Atypical HUS Product Offerings

- **December 2018**: U.S. Approval for PNH
- **1H2019**: File for Approval for atypical HUS
- **1Q2020**: Potential approval for atypical HUS in U.S.
- **2020**: Higher concentration on market
- **2021**: Potential first-to-market SubQ

- **93% PNH Patients prefer ULTOMIRIS**
  - Q8W IV dosing preferred over SubQ delivery options
- **Aligned with patient preference for reduced visit length**
  - Infusion time reduced to ~45 minutes
- **Across indications, provides optionality (e.g., IV, SubQ)**
SOLIRIS® and ULTOMIRIS® Patient Growth in the US

2007-2017
Strong foundation & commercial expertise in PNH & atypical HUS

2017-2019
- Best Alexion launch with SOLIRIS® in gMG
- Potential for SOLIRIS® approval in NMOSD

2020+
Accelerating Neurology growth in gMG and other potential Neurological indications

Ambition for gMG patients to comprise largest SOLIRIS-treated population two years after initial launch
Potential for CAEL-101: Giving Hope for Patients with AL Amyloidosis

Aradhana Sarin, M.D. | Chief Strategy & Business Officer
AL AMYLOIDOSIS: A RARE, FATAL HEMATOLOGIC DISEASE

- AL Amyloidosis is a progressive and typically fatal disease caused by deposition of misfolded immunoglobulin light-chains resulting in severe organ damage
- Multiple organ/tissue involvement; most frequently heart & kidneys
- Often fatal with a median survival of <18 months
- >40% of patients die within one year of diagnosis
  - Degree of cardiac damage can determine survival rate
- Affects >20k patients in US and EU5
- Patients are managed by multifunctional team (i.e. hematologists, oncologists, cardiologists, nephrologists and neurologists)

Typically, chemotherapy regimens used in AL Amyloidosis patients

Chemotherapy targets abnormal plasma cells to shut down future amyloid protein production

But, does not remove previously deposited amyloid protein or reverse organ damage

Only ~20% of patients eligible for ASCT due to severity of disease

- Time to diagnosis is protracted (average ~9 months)
  - At time of diagnosis, most patients have significant fibril deposition in tissues/organs primarily in the heart and kidneys

- Chemotherapy +/- autologous stem cell transplant (ASCT) targets plasma cells the typical treatment approach
  - Only ~20% of patients eligible for ASCT due to severity of disease

- Despite these treatments, survival is poor
CAEL-101 is a chimeric mAb specific to kappa and lambda light chains

- In vivo imaging shows CAEL-101 binding to the heart, kidney, liver, spleen, and other deposits in patients
- Phase 1a/b in AL (n=27) amyloid patients demonstrated a median time to NTproBNP response\(^2\) of 3 weeks vs 10 months with current treatments
- Overall survival at 18.6 months was 93%

### Overall Organ Response Rates\(^1\) After CAEL-101 Phase 1A/1B in AL Amyloid Patients

<table>
<thead>
<tr>
<th></th>
<th>Phase 1a</th>
<th>Phase 1b</th>
<th>Overall</th>
</tr>
</thead>
<tbody>
<tr>
<td>Response</td>
<td>67%</td>
<td>62%</td>
<td>63%</td>
</tr>
<tr>
<td>Stable</td>
<td>33%</td>
<td>39%</td>
<td>37%</td>
</tr>
</tbody>
</table>

### Mean 1.69% improvement in mean GLS score in Phase 1b patients after 12 weeks

1) Includes both cardiac and renal responses 2) Surrogate biomarker of survival in AL amyloidosis
Rare hematologic disease affecting >20k patients in US and EU5

Early proof of concept with CAEL101; Binds to light chains in humans, meaningful cardiac improvement

Working towards Phase 2/3 trial initiation in early 2020
ADVANCING OUR NEUROLOGY BUSINESS

Brian Goff, Chief Commercial Officer
John Orloff, M.D. Head of R&D
Diversifying the Alexion Portfolio

1. Hematology & Nephrology
2. Neurology
3. Metabolic
4. FcRn Portfolio

Financial Ambition to Continue to Deliver Double-Digit Revenue and Non-GAAP EPS Growth
Generalized Myasthenia Gravis

A debilitating, chronic, and progressive autoimmune neuromuscular disease\(^1\)

10-15% of gMG patients fail to respond adequately to or cannot tolerate multiple therapies for gMG and continue to suffer profound muscle weakness and severe disease symptoms that limit function\(^2-4\)

SOME SYMPTOMS OF gMG\(^5-8\)

- Drooping of one or both eyelids
- Blurred or double vision
- Difficulty swallowing or choking
- Shortness of breath
- Slurred speech
- Weakness in the arms, hands, fingers, legs, and neck

SOLIRIS® IS THE FIRST AND ONLY APPROVED COMPLEMENT INHIBITOR FOR gMG\(^9\)

SOLIRIS® QUICK FACTS

- First FDA-approved treatment for patients with gMG in 60+ years in 2017
- Approvals include U.S., EU, Japan, and Canada
- Serving patients who suffer from significant unresolved disease symptoms

---

\(^3\) Howard J. Targeting the Complement System in Refractory Myasthenia Gravis. Supplement to Neurology Reviews February 2016.
In anti-AchR+ gMG, complement damages the NMJ, affecting the ability of the nerves to communicate with the muscles.

**Complement Initiation:**
Antibodies bind to receptors (AchRs) and disrupt nerve-to-muscle communication. They also cause complement to act at the NMJ.

**Ongoing Injury:**
At the NMJ, complement continually injures the muscle surface, which is critical for nerve-to-muscle communication.

**Consequences:**
When the muscle surface is injured, some AchRs are lost, further decreasing nerve-to-muscle communication, which contributes to symptoms of muscle weakness and fatigue.
**MG-302 Interim Data**

- Long-term safety profile of SOLIRIS® consistent with REGAIN
- REGAIN had rigorous enrollment criteria for the most severe patients
  - Patients either failed treatment with at least two ISTs or had failed treatment with at least one IST and required chronic plasma exchange or IVIg
- Improvements seen with SOLIRIS® in REGAIN maintained for up to 3 years
- Positive impact on disease burden:
  - Reduced exacerbation, MG-related hospitalization and rescue therapy
  - Over half of patients achieved a MGFA post-intervention status of minimal manifestations or pharmacologic remission

---

1) Muppidi et al Muscle Nerve 2019 Epub
Note: MG-ADL = Myasthenia Gravis-Activities of Daily Living Profile; MGFA = Myasthenia Gravis Foundation of America
Expect gMG to be largest U.S. patient volume indication for SOLIRIS® by YE19

US gMG Patients on SOLIRIS®

- December 31, 2017: 43
- March 31, 2018: 194
- June 30, 2018: 375
- September 30, 2018: 560
- December 31, 2018: 788
- March 19, 2019*: 948

*Not full quarter
EXPANDING gMG PORTFOLIO

gMG Portfolio Strategy

- Expanding treatment options for patients with gMG with ULTOMIRIS® IV and SubQ administration
  - Initiating Phase 3 trial in 2019
  - No prior failure of IST therapies required for enrollment; MG-ADL score of ≥ 6 required
- Aligned with broader strategy in Neurology and transition from a focus on ultra-rare to rare diseases
- Established proof of concept & mechanism established with FcRn; plan to advance ALXN1830 into gMG by YE19
NMOSD Is a Complement-Mediated Disease\textsuperscript{1,2}

- Complement activation triggered by the binding of an IgG autoantibody to aquaporin 4 (AQP4) on the astrocyte membrane causing CNS inflammation and demyelination
- Complement activity attracts leukocytes, leading to degranulation and astrocyte destruction

NMOSD is Characterized By Step-wise Deterioration Following Each Attack

Relapses can be devastating for patients, with unpredictable & cumulative functional decline

Each attack can lead to cognitive worsening, encephalopathy, seizures, pain, paralysis, and vision loss, blindness or death

Phase 3 PREVENT study results

98% of patients relapse free at 48 weeks

- Relapse prevention is the goal of treatment
- Remarkable strength of NMOSD clinical data with 94.2% reduction in risk for relapse at 48 weeks

~96% of patients relapse free at 3 years

Strong synergies in place with expanded existing neurology infrastructure

- SOLIRIS® NMOSD sBLA accepted in US, EU and Japan
- Granted priority review in US based on strength of Ph3 PREVENT clinical data
  - PDUFA date June 28, 2019
- Training and launch excellence planning across commercial and medical teams
- Upon approval, dedicated customer-facing teams, OneSource™ case managers, as well as payer and market access

*In the PREVENT Phase 3 trial, both Soliris and Placebo arm allowed for concomitant use of immunosuppressant therapy (IST)*
ULTOMIRIS Phase 3 NMOSD Study Design

- Leverage neurology footprint and PREVENT results to drive trial recruitment and optimize product profile
- Phase 3 single arm estimation study in adult patients (n = ~65) leveraging SOLIRIS® PREVENT results as contemporaneous control
- Plan to initiate in 4Q19 with bridging to ULTOMIRIS SC pending regulatory feedback on study design & bridging approach
MICHAELE LEVY, MD
Associate Professor, Department of Neurology
Massachusetts General Hospital

• Principal Investigator, PREVENT Phase 3

Panel Moderator:
Laura Gault, MD, PhD
Neurology Clinical Development Head
Complement confirmed to play a key role in both the CNS and Neuromuscular Junction with SOLIRIS® in gMG and NMOSD

### Scientific rationale supports potential role of complement, including MAC deposition in ALS and elevation of C3 and C4 in PPMS patients

<table>
<thead>
<tr>
<th><strong>Amyotrophic Lateral Sclerosis (ALS)</strong></th>
<th><strong>Primary Progressive Multiple Sclerosis (PPMS)</strong></th>
</tr>
</thead>
<tbody>
<tr>
<td>- Neurodegenerative disease characterized by motor neuron degeneration leading to progressive muscle weakness</td>
<td>- Progressive, worsening neurologic disease characterized by decreased mobility, functional impairment, cognitive changes</td>
</tr>
<tr>
<td>- High mortality rate 3-5 years post-diagnosis</td>
<td>- Estimated 30-40K addressable population in US, EU5, and Japan</td>
</tr>
<tr>
<td>- Estimated 15-20K addressable population in US, EU5, and Japan</td>
<td>- 15% of MS patients diagnosed with PPMS</td>
</tr>
<tr>
<td>- Plan to initiate POC clinical trial in 2019</td>
<td>- Plan to explore role of complement in clinical study in 2019</td>
</tr>
</tbody>
</table>
Scientific Rationale

- MAC deposition in motor end plate suggests a role of complement
- Elevated complement activation products in ALS serum and CSF

Phase 2/3 Adaptive Trial Design

- Primary endpoint measures $\Delta$ from baseline in revised ALSFRS-R\textsuperscript{1}
- Plans to initiate in 4Q19

FACTS ABOUT ALS

- 5,000 people are diagnosed per year
- 10% of cases are inherited through a mutated gene
- 2-5 YEARS is the average life expectancy
- Every 90 MINUTES someone is diagnosed and someone passes away
- 90% of cases occur without family history
- $\$250,000 is the estimated out-of-pocket cost for caring for a person with ALS
- Only 4 DRUGS are currently approved by the U.S. FDA to treat ALS (Riluzole, Mestinon, Radicava, and Tigril)
- $\$2 BILLION is the estimated cost to develop a drug to slow or stop the progression of ALS

1. ALS Functional Rating Scale
Building on Rare Disease Expertise to Broaden Neurology Growth Opportunity

October 2017
SOLIRIS® approved with broad label for gMG

December 2018
SOLIRIS® in gMG best Alexion launch with 788 patients on therapy

Year End 2019
Ambition for neurology to be largest US franchise

2017 2018 2019 2020 2021+

September 2018
Positive SOLIRIS® Phase 3 data in NMOSD

February 2019
Granted Priority Review for SOLIRIS® in NMOSD PDUFA June 28th

Year End 2019
Initiate Phase 3 trials for ULTOMIRIS® in gMG and NMOSD and clinical trials in ALS and PPMS
Initiate pivotal trial for ALXN1830 in gMG

2021+
Serving broad patient populations across numerous indications
Convenient and long-acting dosing potential

Neurology franchise represents multi-billion dollar revenue potential
METABOLICS: TRANSFORMING LIVER & BONE DISEASE

Brian Goff, Chief Commercial Officer
John Orloff, M.D. Head of R&D
Diversifying the Alexion Portfolio

1. Hematology & Nephrology
2. Neurology
3. Metabolics
4. FcRn Portfolio

Financial Ambition to Continue to Deliver Double-Digit Revenue and Non-GAAP EPS Growth
SHAWN’S JOURNEY WITH HPP
SHAWN’S JOURNEY WITH HPP
SOME SYMPTOMS OF HPP\textsuperscript{1-5}

- Hypomineralization of bone
- Fractures and skeletal abnormalities
- Muscle weakness
- Seizures in perinatal/infantile forms
- Bone/joint/muscle pain
- Developmental delays/impaired mobility
- Respiratory failure due to rachitic chest, leading to premature death in infants

QUICK FACTS

- It is estimated that in the United States, there are only approximately 1,300 people who have HPP
- STRENSIQ\textsuperscript{\textregistered} first approved in 2015
- STRENSIQ\textsuperscript{\textregistered} treatment demonstrated a survival rate of 97% in patients with perinatal/infantile-onset HPP compared to 42% in untreated historical controls at one year in the clinical development program\textsuperscript{7}

Hypophosphatasia

is a progressive, systemic, inherited, potentially life-threatening metabolic disorder characterized by low alkaline phosphatase enzyme activity

Without treatment, only

27%

of infants with HPP symptom onset in the first 6 months of life survive beyond 5 years\textsuperscript{6}

HPP is an ultra-rare disease affecting hundreds of patients in the United States

- Diagnose patients earlier in their disease
- Efficient targeting of a broad range of specialties that treat patients with HPP
- Expanded Laboratory Education (CALIPER Initiative)
- Innovative and proactive contracting for access sustainability
EXPANDED EFFORT TO EDUCATE ON APPROPRIATE DIAGNOSTIC RANGES

Many laboratories use only adult reference ranges for alkaline phosphatase (ALP) which is indicated as being low below a value of 40

- 71% of pediatric cases are not diagnosed due to incorrect reference ranges

Initiative to drive laboratory adoption of pediatric age- and sex-adjusted reference (CALIPER) ranges

Pediatric HPP patients potentially missed due to use of adult ALP reference ranges

1) ALP values from CALIPER, which has established age- and sex-specific reference intervals using blood samples from more than 9700 healthy children and adolescents. 2) Abbott Clinical Chemistry Architect System ALP reference range lower limit of normal for males >20 and females >15 years of age.
**Lysosomal Acid Lipase Deficiency (LAL-D)**

is a life-threatening genetic disease with progressive multi-organ damage leading to premature death\(^1\)

**Without treatment, median age of death is**

**3.7 Months**

In LAL-D patients who experience symptoms in infancy\(^4,5\)

---

**SOME SYMPTOMS OF LAL-D\(^2,3\)**

- Multi-organ damage
- Liver damage including fibrosis, cirrhosis, and failure
- Failure to thrive and premature death
- Cardiovascular disease manifestations including dyslipidemia, accelerated atherosclerosis, coronary artery disease

---

**KANUMA® QUICK FACTS**

- KANUMA® treatment demonstrated a survival rate of 67% in infants with LAL-D vs. 0% in untreated historical controls at one year in the clinical development program\(^6\)
- Continuing to identify new patients
- Potential future strong call point synergies with Wilson Disease

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ALXN1840: A NOVEL, POTENTIAL FIRST-IN-CLASS SMALL MOLECULE FOR WILSON DISEASE

SOME SYMPTOMS OF WILSON DISEASE
- Jaundice & fluid retention
- Confusion, psychosis, and psychiatric disorders
- Increased risk of cirrhosis, liver failure, and liver cancer
- Fatigue, pain and swelling
- Vomiting and gastrointestinal bleeding
- Neurological morbidity

ABOUT ALXN1840
- Ongoing phase 3 trial powered for superiority; data expected 1H21
- Differentiated product profile compared to current standard of care
- Potential for first line treatment utilization

ALXN1840: A Differentiated Product Profile with Unique MOA

1. High affinity to Cu
2. Specific to Cu
3. Forms stable tripartite complexes with proteins
4. Excretion of Cu through bile into feces
5. Simplified dosing regimen

- 10,000-fold higher affinity for Cu than chelators, allowing for removal from intracellular stores in the liver
- Specifically binds Cu, not other metals (Zn, Fe, Ca, Mn, Mg) typically associated with treatment side effects
- Safe Cu transport in the blood, reducing the risk of drug induced neurological and psychiatric deficits
- Excretes excess Cu via natural route limiting potential nephrotoxicity
- Oral, once daily dosing and rapid onset of action
Improvements seen at week 24 were maintained through extension phase.

ALXN1840 reduced NCC_cor1 levels by similar extents in patients with and without cirrhosis, from baseline to week 24.

The Unified Wilson’s Disease Rating Scale (UWDRS) was used to assess patient-reported disability² and clinician-rated neurological status³.

Improvements occurred during the core study⁴ and to week 48, regardless of cirrhosis status.

ALXN1840 stabilized and reduced disability and improved neurological status.

1 NCC levels were not corrected at baseline, as no ALXN1840 had been received; ²Score range, 0–40; ³Score range, 0–143; ⁴Data have been published previously for the whole study population during the core study: Weiss KH et al. Lancet Gastroenterol; b/d data from an early termination visit for one patient who had discontinued from the core study was included at this time point; BL, baseline; SEM, standard error of the mean; ULN, upper limit of normal reference range (2.3 μmol/L); A. Czlokowska et al., Presented at European Academy of Neurology – European J Neurol, 2018 (data to 72 wks).
FOCuS: Phase 3 Trial Powered for Superiority vs SoC

- **Cohort 1**
  - Pre-treated with SoC >28 days
  - 75% Patients

- **Cohort 2**
  - Treatment naïve or minimally pre-treated with SoC ≤ 28 days
  - 25% Patients

- **2:1 Randomization**
  - Switch to ALXN1840
  - Stay on SoC
  - Start on ALXN1840
  - Start or Stay on SoC

- **Extension Phase**
  - All Patients Treated with ALXN1840

- **48 Weeks**

- **Until Approval**
MICHAEL SCHILSKY, MD
Medical Director, Adult Liver Transplant at Yale-New Haven Transplantation Center

- Principal Investigator, ALXN1840 Clinical Program

Panel Moderator:
Michele Mercuri, MD, PhD
Metabolics Clinical Development Head
FcRn PORTFOLIO:
Rare Autoimmune Diseases
John Orloff, M.D. Head of R&D
Diversifying the Alexion Portfolio

1. Hematology & Nephrology
2. Neurology
3. Metabolic
4. FcRn Portfolio

Financial Ambition to Continue to Deliver Double-Digit Revenue and Non-GAAP EPS Growth
Neonatal Fc receptor (FcRn) is a protein that binds all IgG subclasses under acidic pH

FcRn is involved in regulating IgG turnover:
- FcRn binds to IgG in acidic endosomes, recycles it to cell surface where IgG is released into circulation
- FcRn activity prevents IgG from undergoing lysosomal degradation and contributes to its long half-life

Disrupting the IgG-FcRn interaction increases the clearance of IgG which is believed to reduce levels of pathogenic autoantibodies

FcRn inhibitors have potential to become a new generation of therapeutics for IgG-mediated diseases
Multiple Clinical Stage Programs

- Broad applicability across numerous rare autoimmune diseases
- Strong strategic fit for focus on rare diseases with high unmet need in hematology, neurology, and other TAs

<table>
<thead>
<tr>
<th>Development Summary</th>
<th>WAIHA</th>
<th>Phase 1/2</th>
<th>Phase 3</th>
</tr>
</thead>
<tbody>
<tr>
<td>ALXN1830</td>
<td></td>
<td>Data 1H19</td>
<td></td>
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<tr>
<td>ALXN1830</td>
<td></td>
<td>Planning to initiate in 2019</td>
<td></td>
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<tr>
<td>gMG</td>
<td>ALXN1830</td>
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<td>SAD/MAD</td>
<td></td>
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<tr>
<td>ABY-039</td>
<td>ALXN1830</td>
<td>Planning to initiate in 2019</td>
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</table>

- Additional indications in clinical trials in 2020
- Data from 1830 Phase 1/2 studies in 2019

ALXN1830

- A humanized IgG4 monoclonal antibody
- **Proof of concept established** in Phase 1b/2a
- **High specificity** to IgG and **no reduction in albumin** observed
- **Rapid onset of action** in early studies
- Anticipate Q2W IV Dosing in Phase 3 Studies
- Plans to pursue **SubQ formulation**

**ABY-039**

- Collaboration with Affibody
- A high affinity protein ligand with extended half-life conferred by albumin-binding domain
- Expanding FcRn opportunity with potential for **best-in-class SubQ dosing**
- Ongoing Phase 1 SAD/MAD clinical trial
Significant Need for Effective Therapies

- ~65k patients in the US and EU5
  - 1/3 continue to have active disease despite treatment
- No approved treatment options
- Strong strategic fit with existing hematology franchise
- ALXN1830 remains the first and only anti-FcRn in development for warm autoimmune hemolytic anemia
- Complications can include:
  - Weakness
  - Fatigue
  - Dyspnea
  - Syncope
  - Angina
  - Tachycardia
  - Heart Failure
  - Hepatomegaly
  - Splenic Enlargement

**ALXN1830 has potential for first-line treatment utilization**

- Future Treatment Paradigm (Illustrative)
  - Oral or IV Corticosteroids ± Transfusions
  - Rituximab or ALXN1830 or Splenectomy
  - IVlg or Immuno-Suppressant or Rituximab or Splenectomy

**Phase 3 Study Design**

- WAIHA Patients
  - Randomized 1:1
  - ALXN1830 (n=25) + SoC
  - Open Label Extension
  - PBO (n=25) + SoC
  - 6 month treatment period
### Expanding Alexion Treatment Options for Patients with gMG

- Established proof-of-concept for anti-FcRn mechanism in gMG
- ALXN1830 will complement Alexion’s C5 gMG portfolio
- Aligned with broader company development efforts in Neurology
- Opportunity to leverage gMG development, regulatory and commercial expertise in potential earlier-line therapy

### ALXN1830 Phase 3 Trial Design

- Double-blind, placebo-controlled trial to start 4Q19
  - Adults with mild-to-severe gMG
  - Expanding addressable population with AcHR+, MuSK+, LRP4+ patients
- Primary endpoint of Δ in MG-ADL
- Leverage neurology footprint to drive trial recruitment and optimize product profile

### ALXN1830 Dose Optimization

- Anticipate Q2W IV Dosing in Phase 3 Studies
- Plan to pursue SubQ formulation

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Note: ULTOMIRIS® is not approved to treat patients with gMG
**ABY-039: Potential for Optimized Subcutaneous Dosing**

**Small Size (~19 kDa)**
- High-dose, **low-volume subQ dosing**
- Optimal for at **home use and self-administration**
- Up to **10x higher dose per injection** volume compared to mAbs

**Potent Binding Affinity**
- **Rapid onset**
- Surface optimized designed proteins with **high binding affinity**
- Efficient autoantibody reduction

**Long Half-Life**
- Long duration of action
- **Half-life of ABY-039 significantly longer than a standard anti-FcRn antibody** due to the Albumod technology and albumin tether
- Potential for **longer-acting** subQ dosing
COMPLEMENT RESEARCH EXPERTISE:
Driving the Next Generation of Treatments
Sharon Barr, Ph.D. | Head of Research
A RELENTLESS PURSUIT OF INNOVATION

- Research concentrated in New Haven with expertise in Ab engineering
- Numerous promising internal projects and collaborations with trusted partners
- Innovative assays, novel biomarkers, and new diagnostics to support efficient clinical trials
- Robust, diverse complement pipeline to deliver sustainable value
- Multiple INDs over the coming years

Goal to create novel therapeutics to treat patients with rare diseases, leveraging industry-leading expertise in complement biology
- Complement is a master sensor that discriminates between foreign or altered and healthy cell surfaces
- Rationale for complement inhibition across multiple rare disease indications and therapeutic areas
- Complexity of complement biology allows for multiple targeting approaches
- Pursuing novel molecules and targets across terminal, lectin, and alternative pathways
**ALXN1720: A NOVEL ANTI-C5/ANTI-ALBUMIN BISPESIFIC**

- Bi-specific mini-body that binds and prevents activation of human C5
- Specifically designing for long-acting, SubQ dosing:
  - 25 kDa size with potential for auto-injector or pre-filled syringe
  - Long half-life by binding to human serum albumin
- On track for first-in-human study in 2019

**Plans to develop for multiple new indications and therapeutic areas (e.g., Nephrology)**
Peripheral C6 crosses the blood brain barrier to enter the CNS

Circulating C6 in CNS leads to formation of MAC which can cause neurodegeneration

CP010 binds to peripheral C6 to reduce the levels of C6 in the CNS and decrease MAC formation

- Expands our complement franchise with a novel asset (CP010) addressing neurological disorders
- Membrane attack complex (MAC) formation in central nervous system is dependent upon peripheral C6 as evidence suggests C6 is not produced in the CNS
- CP010 binds to peripheral C6 to decrease the level of C6 in the CNS to achieve effective inhibition of MAC formation
Innovative RNAi technology with potential for multiple targets

Clinical proof-of-concept in Primary Hyperoxaluria

Broad therapeutic applicability in complement-mediated diseases

Low volume (up to 2 mL) injection with long duration of effect supporting monthly-to-quarterly dosing

Theoretical off-target effects from breakdown products are minimized through chemical modifications

- GALXC’s RNAi platform silences hepatic gene expression
- The GalNAc sugars enable specific delivery to hepatocytes through binding to asialoglycoprotein receptor (ASGPR)
PARTNERSHIP TO DEVELOP PEPTIDE THERAPEUTICS

- Adds additional preclinical assets to complement pipeline
- Innovative technology platform already commercialized for other targets
- Peptide therapies offer a highly selective targeted approach with high potency, low concentrations and small size for ease of administration
- Potential for multiple targets within the complement cascade
- Broad therapeutic applicability in complement mediated diseases

Cutting Edge Peptide Drug Candidates
- Physical Stability
- Chemical Stability
- Solubility
- Potency
2019 OBJECTIVES

1. ULTOMIRIS™ Conversion in PNH; aHUS filing
2. Accelerate Neurology Portfolio
3. Grow our Metabolics Portfolio
4. Execute and Expand the Pipeline
5. Deliver on Financial Ambitions
Diversifying the Alexion Portfolio

1. **Hematology & Nephrology**
   - Facilitate PNH Conversion to ULTOMIRIS™, 1
   - Report Phase 3 aHUS data
   - File aHUS in US, EU & Japan

2. **Neurology**
   - Launch SOLIRIS® in NMOSD in the US 2
   - Grow SOLIRIS® in gMG
   - Initiate ULTOMIRIS Phase 3 studies in gMG & NMOSD

3. **Metabolics**
   - Execute Phase 3 ALXN1840 superiority trial
   - Expand Metabolic Portfolio
   - Grow STRENSIQ® & KANUMA®

4. **FcRn Portfolio**
   - ALXN1830 Phase 1/2 data in WAIHA read-out
   - Dose-optimization data read-out
   - Initiate two ALXN1830 pivotal trials

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Financial Ambition to Continue to Deliver Double-Digit Revenue and Non-GAAP EPS Growth

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1 Ex-US upon regulatory approval
2 Upon regulatory approval
**Strategy for Durable, Long-Term Growth**

**Vision & Strategy** | Ludwig Hansson, PhD, CEO

Business Development

Organic Clinical Pipeline

Internal Complement Research

- Hematology & Nephrology
- Neurology
- Metabolics
- FcRn Portfolio